



IFWO

RAW SEQUENCE LISTING

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:50

Input Set : N:\Cr3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

1 <110> APPLICANT: KAKKIS, EMIL D.
 2 <120> TITLE OF INVENTION: METHODS FOR TREATING DISEASES CAUSED BY DEFICIENCIES OF
 3 RECOMBINANT ALPHA-L-IDURONDINASE
 4 <130> FILE REFERENCE: 008000051CNUS01
 5 <140> CURRENT APPLICATION NUMBER: US/10/722,371
 6 <141> CURRENT FILING DATE: 2003-11-24
 7 <150> PRIOR APPLICATION NUMBER: US/09/993,038
 8 <151> PRIOR FILING DATE: 2001-11-13
 9 <150> PRIOR APPLICATION NUMBER: 09/711,205
 10 <151> PRIOR FILING DATE: 2000-11-09
 11 <150> PRIOR APPLICATION NUMBER: 09/439,923
 12 <151> PRIOR FILING DATE: 1999-11-12
 13 <160> NUMBER OF SEQ ID NOS: 2
 14 <170> SOFTWARE: FastSEQ for Windows Version 3.0
 16 <210> SEQ ID NO: 1
 17 <211> LENGTH: 6200
 18 <212> TYPE: DNA
 19 <213> ORGANISM: Homo sapiens
 20 <220> FEATURE:
 21 <221> NAME/KEY: CDS
 22 <222> LOCATION: (1558)...(3510)
 23 <400> SEQUENCE: 1

24	gacggatcgg	gagatctccc	gatccccctat	ggtegactct	cagtacaatc	tgctctgatg	60
25	ccgcatagtt	aagccagtat	ctgctccctg	cttgtgtgtt	ggaggtcgct	gagtagtgcg	120
26	cgagcaaaat	ttaagctaca	acaaggcaag	gcttgaccga	caattgcatg	aagaatctgc	180
27	ttagggttag	gcgttttgcg	ctgcttcgcg	atgtacgggc	cagatatacg	cgttgacatt	240
28	gattattgac	tagttattaa	tagtaatcaa	ttacggggtc	attagttcat	agcccatata	300
29	tggagttccg	cgttacataa	cttacggtaa	atggcccggc	tggctgaccg	cccaacgacc	360
30	cccgcctatt	gacgtcaata	atgacgtatg	ttcccatagt	aacgcccaata	gggactttcc	420
31	attgacgtca	atgggtggac	tattttacgg	aaactgcccc	cttggcagta	catcaagtgt	480
32	atcatatgcc	aagtaacgcc	cctattgacg	tcaatgacgg	taaatggccc	gcctggcatt	540
33	atgccacgta	catgacctta	tgggactttc	ctacttgcca	gtacatctac	gtattagtca	600
34	tcgctattac	catggtgatg	cgtttttggc	agtacatcaa	tgggcgtgga	tagcggtttg	660
35	actcacgggg	atttccaagt	ctccacccca	ttgacgtcaa	tgggagtttg	ttttggcacc	720
36	aaaatcaacg	ggactttcca	aaatgtcgta	acaactccgc	cccattgacg	caaatgggcg	780
37	gtaggcgtgt	acgggtggag	gtctatataa	gcagagctct	ctggctaact	agagaaccca	840
38	ctgcttaact	ggcttatcga	aattaatacg	actcactata	gggagaccca	agcttcgcag	900
39	aattcctgcg	gctgctacag	tgtgtccagc	gtcctgacct	gctgtgctga	gcgctggaac	960
40	agtggcgcat	cattcaagtg	cacagttacc	catcctgagt	ctggcacctt	aactggcaca	1020
41	attgccaaaag	tcacaggtga	gctcagatgc	ataccaggac	attgtatgac	gttccttgct	1080
42	cacatgcctg	ctttcttcct	ataatacaga	tgtctcaacta	actgctcatg	tccttatatc	1140
43	acagagggaa	attggagcta	tctgaggaac	tgcccagaag	ggaagggcag	aggggtcttg	1200
44	ctctccttgt	ctgagccata	actcttcttt	ctaccttcca	gtgaacacct	tcccacccca	1260

RAW SEQUENCE LISTING

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:50

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

```

45 ggtccacctg ctaccgccgc cgtcggagga gctggccctg aatgagctct tgtccctgac 1320
46 atgcctggtg cgagctttca accctaaaga agtgctggtg cgatggctgc atggaaatga 1380
47 ggagctgtcc ccagaaagct acctagtgtt tgagccccta aaggagccag gcgagggagc 1440
48 caccacctac ctggtgacaa gcgtgttgcg tgtatcagct gaaagcttga tatcgaattc 1500
49 cggaggcgga accggcagtg cagcccgaag ccccgagtc cccgagcacg cgtggcc atg 1560
50 Met
51 1
52 cgt ccc ctg cgc ccc cgc gcc gcg ctg ctg gcg ctc ctg gcc tcg ctc 1608
53 Arg Pro Leu Arg Pro Arg Ala Ala Leu Leu Ala Leu Leu Ala Ser Leu
54 5 10 15
55 ctg gcc gcg ccc ccg gtg gcc ccg gcc gag gcc ccg cac ctg gtg cat 1656
56 Leu Ala Ala Pro Pro Val Ala Pro Ala Glu Ala Pro His Leu Val His
57 20 25 30
58 gtg gac gcg gcc cgc gcg ctg tgg ccc ctg cgg cgc ttc tgg agg agc 1704
59 Val Asp Ala Ala Arg Ala Leu Trp Pro Leu Arg Arg Phe Trp Arg Ser
60 35 40 45
61 aca ggc ttc tgc ccc ccg ctg cca cac agc cag gct gac cag tac gtg 1752
62 Thr Gly Phe Cys Pro Pro His Ser Gln Ala Asp Gln Tyr Val
63 50 55 60 65
64 ctc agc tgg gac cag cag ctc aac ctc gcc tat gtg ggc gcc gtc cct 1800
65 Leu Ser Trp Asp Gln Gln Leu Asn Leu Ala Tyr Val Gly Ala Val Pro
66 70 75 80
67 cac cgc ggc atc aag cag gtc cgg acc cac tgg ctg ctg gag ctt gtc 1848
68 His Arg Gly Ile Lys Gln Val Arg Thr His Trp Leu Leu Glu Leu Val
69 85 90 95
70 acc acc agg ggg tcc act gga cgg ggc ctg agc tac aac ttc acc cac 1896
71 Thr Thr Arg Gly Ser Thr Gly Arg Gly Leu Ser Tyr Asn Phe Thr His
72 100 105 110
73 ctg gac ggg tac ctg gac ctt ctc agg gag aac cag ctc ggg ttt gag 1944
74 Leu Asp Gly Tyr Leu Asp Leu Leu Arg Glu Asn Gln Leu Gly Phe Glu
75 115 120 125
76 ctg atg ggc agc gcc tcg ggc cac ttc act gac ttt gag gac aag cag 1992
77 Leu Met Gly Ser Ala Ser Gly His Phe Thr Asp Phe Glu Asp Lys Gln
78 130 135 140 145
79 cag gtg ttt gag tgg aag gac ttg gtc tcc agc ctg gcc agg aga tac 2040
80 Gln Val Phe Glu Trp Lys Asp Leu Val Ser Ser Leu Ala Arg Arg Tyr
81 150 155 160
82 atc ggt agg tac gga ctg gcg cat gtt tcc aag tgg aac ttc gag acg 2088
83 Ile Gly Arg Tyr Gly Leu Ala His Val Ser Lys Trp Asn Phe Glu Thr
84 165 170 175
85 tgg aat gag cca gac cac cac gac ttt gac aac gtc tcc atg acc atg 2136
86 Trp Asn Glu Pro Asp His His Asp Phe Asp Asn Val Ser Met Thr Met
87 180 185 190
88 caa ggc ttc ctg aac tac tac gat gcc tgc tcg gag ggt ctg cgc gcc 2184
89 Gln Gly Phe Leu Asn Tyr Tyr Asp Ala Cys Ser Glu Gly Leu Arg Ala
90 195 200 205
91 gcc agc ccc gcc ctg cgg ctg gga ggc ccc ggc gac tcc ttc cac acc 2232
92 Ala Ser Pro Ala Leu Arg Leu Gly Gly Pro Gly Asp Ser Phe His Thr
93 210 215 220 225

```

RAW SEQUENCE LISTING

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:50

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

94	cca ccg cga tcc ccg ctg agc tgg ggc ctc ctg cgc cac tgc cac gac	2280
95	Pro Pro Arg Ser Pro Leu Ser Trp Gly Leu Leu Arg His Cys His Asp	
96	230 235 240	
97	ggt acc aac ttc ttc act ggg gag gcg ggc gtg cgg ctg gac tac atc	2328
98	Gly Thr Asn Phe Phe Thr Gly Glu Ala Gly Val Arg Leu Asp Tyr Ile	
99	245 250 255	
100	tcc ctc cac agg aag ggt gcg cgc agc tcc atc tcc atc ctg gag cag	2376
101	Ser Leu His Arg Lys Gly Ala Arg Ser Ser Ile Ser Ile Leu Glu Gln	
102	260 265 270	
103	gag aag gtc gtc gcg cag cag atc cgg cag ctc ttc ccc aag ttc gcg	2424
104	Glu Lys Val Val Ala Gln Gln Ile Arg Gln Leu Phe Pro Lys Phe Ala	
105	275 280 285	
106	gac acc ccc att tac aac gac gag gcg gac ccg ctg gtg ggc tgg tcc	2472
107	Asp Thr Pro Ile Tyr Asn Asp Glu Ala Asp Pro Leu Val Gly Trp Ser	
108	290 295 300 305	
109	ctg cca cag ccg tgg agg gcg gac gtg acc tac gcg gcc atg gtg gtg	2520
110	Leu Pro Gln Pro Trp Arg Ala Asp Val Thr Tyr Ala Ala Met Val Val	
111	310 315 320	
112	aag gtc atc gcg cag cat cag aac ctg cta ctg gcc aac acc acc tcc	2568
113	Lys Val Ile Ala Gln His Gln Asn Leu Leu Leu Ala Asn Thr Thr Ser	
114	325 330 335	
115	gcc ttc ccc tac gcg ctc ctg agc aac gac aat gcc ttc ctg agc tac	2616
116	Ala Phe Pro Tyr Ala Leu Leu Ser Asn Asp Asn Ala Phe Leu Ser Tyr	
117	340 345 350	
118	cac ccg cac ccc ttc gcg cag cgc acg ctc acc gcg cgc ttc cag gtc	2664
119	His Pro His Pro Phe Ala Gln Arg Thr Leu Thr Ala Arg Phe Gln Val	
120	355 360 365	
121	aac aac acc cgc ccg ccg cac gtg cag ctg ttg cgc aag ccg gtg ctc	2712
122	Asn Asn Thr Arg Pro Pro His Val Gln Leu Leu Arg Lys Pro Val Leu	
123	370 375 380 385	
124	acg gcc atg ggg ctg ctg gcg ctg ctg gat gag gag cag ctc tgg gcc	2760
125	Thr Ala Met Gly Leu Leu Ala Leu Leu Asp Glu Glu Gln Leu Trp Ala	
126	390 395 400	
127	gaa gtg tgc cag gcc ggg acc gtc ctg gac agc aac cac acg gtg ggc	2808
128	Glu Val Ser Gln Ala Gly Thr Val Leu Asp Ser Asn His Thr Val Gly	
129	405 410 415	
130	gtc ctg gcc agc gcc cac cgc ccc cag ggc ccg gcc gac gcc tgg cgc	2856
131	Val Leu Ala Ser Ala His Arg Pro Gln Gly Pro Ala Asp Ala Trp Arg	
132	420 425 430	
133	gcc gcg gtg ctg atc tac gcg agc gac gac acc cgc gcc cac ccc aac	2904
134	Ala Ala Val Leu Ile Tyr Ala Ser Asp Asp Thr Arg Ala His Pro Asn	
135	435 440 445	
136	cgc agc gtc gcg gtg acc ctg cgg ctg cgc ggg gtg ccc ccc ggc ccg	2952
137	Arg Ser Val Ala Val Thr Leu Arg Leu Arg Gly Val Pro Pro Gly Pro	
138	450 455 460 465	
139	ggc ctg gtc tac gtc acg cgc tac ctg gac aac ggg ctc tgc agc ccc	3000
140	Gly Leu Val Tyr Val Thr Arg Tyr Leu Asp Asn Gly Leu Cys Ser Pro	
141	470 475 480	
142	gac ggc gag tgg cgg cgc ctg ggc cgg ccc gtc ttc ccc acg gca gag	3048

RAW SEQUENCE LISTING

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:50

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

143	Asp Gly Glu Trp Arg Arg Leu Gly Arg Pro Val Phe Pro Thr Ala Glu	
144	485 490 495	
145	cag ttc cgg cgc tag cgc gcg gct gag gac ccg gtg gcc gcg gcg ccc	3096
146	Gln Phe Arg Arg * Arg Ala Ala Glu Asp Pro Val Ala Ala Ala Pro	
147	500 505 510	
148	cgc ccc tta ccc gcc ggc ggc cgc ctg agg ctg cgc ccc gcg ctg cgg	3144
149	Arg Pro Leu Pro Ala Gly Gly Arg Leu Arg Leu Arg Pro Ala Leu Arg	
150	515 520 525	
151	ctg ccg tcg ctt ttg ctg gtg cac gtg tgt gcg cgc ccc gag aag ccg	3192
152	Leu Pro Ser Leu Leu Leu Val His Val Cys Ala Arg Pro Glu Lys Pro	
153	530 535 540	
154	ccc ggg cag gtc acg cgg ctc cgc gcc ctg ccc ctg acc caa ggg cag	3240
155	Pro Gly Gln Val Thr Arg Leu Arg Ala Leu Pro Leu Thr Gln Gly Gln	
156	545 550 555 560	
157	ctg gtt ctg gtc tgg tcg gat gaa cac gtg ggc tcc aag tgc ctg tgg	3288
158	Leu Val Leu Val Trp Ser Asp Glu His Val Gly Ser Lys Cys Leu Trp	
159	565 570 575	
160	aca tac gag atc cag ttc tct cag gac ggt aag gcg tac acc ccg gtc	3336
161	Thr Tyr Glu Ile Gln Phe Ser Gln Asp Gly Lys Ala Tyr Thr Pro Val	
162	580 585 590	
163	agc agg aag cca tcg acc ttc aac ctc ttt gtg ttc agc cca gac aca	3384
164	Ser Arg Lys Pro Ser Thr Phe Asn Leu Phe Val Phe Ser Pro Asp Thr	
165	595 600 605	
166	ggt gct gtc tct ggc tcc tac cga gtt cga gcc ctg gac tac tgg gcc	3432
167	Gly Ala Val Ser Gly Ser Tyr Arg Val Arg Ala Leu Asp Tyr Trp Ala	
168	610 615 620	
169	cga cca ggc ccc ttc tcg gac cct gtg ccg tac ctg gag gtc cct gtg	3480
170	Arg Pro Gly Pro Phe Ser Asp Pro Val Pro Tyr Leu Glu Val Pro Val	
171	625 630 635 640	
172	cca aga ggg ccc cca tcc ccg ggc aat cca tgagcctgtg ctgagcccca	3530
173	Pro Arg Gly Pro Pro Ser Pro Gly Asn Pro	
174	645 650	
175	gtggggttgca cctccaccgg cagtcagcga gctggggctg cactgtgccc atgctgccct	3590
176	cccatcaccc cctttgcaat atatttttat atttttaaaaa aaaaaaaaaa aaaaaaaaaa	3650
177	aaaaaaaaaa aaaaaaaaaa aaaaaaaaaa aaaaaaaaaa aaaaaaaaaa aattcctgca	3710
178	gcccggggga tccactagtt ctgaggggcc cgtttaaacc cgctgatcag cctcgactgt	3770
179	gccttctagt tgccagccat ctgttggttg cccctcccc gtgccttccct tgaccctgga	3830
180	aggtgccact cccactgtcc tttcctaata aaatgaggaa attgcatcgc attgtctgag	3890
181	taggtgtcat tctattctgg ggggtggggg ggggcaggac agcaaggggg aggattggga	3950
182	agacaatagc aggcattgctg gggatgcggg gggctctatg gcttctgagg cggaagaac	4010
183	cagctggggc tcgagagctt ggcgtaatca tggctatagc tgtttcctgt gtgaaattgt	4070
184	tatccgctca caattccaca caacatacga gccggaagca taaagtgtaa agcctggggg	4130
185	gcctaattgag tgagctaact cacattaatt gcgttgcgct cactgcccgc tttccagtcg	4190
186	ggaaacctgt cgtgccagct gcattaatga atcgcccaac gcgcggggag aggcgggttg	4250
187	cgtattgggc gctcttcgcg ttcctcgctc actgactcgc tcgctcggt cgctcggtg	4310
188	cggcgagcgg tatcagctca ctcaaaggcg gtaatacggg tatccacaga atcaggggat	4370
189	aacgcaggaa agaacatgtg agcaaaaggc cagcaaaagg ccaggaaccg taaaaaggcc	4430
190	gcgttgctgg cgtttttcca taggctccgc cccctgacg agcatcaca aaatcgacg	4490
191	tcaagtacga ggtggcgaaa cccgacagga ctataaagat accaggcggt tccccctgga	4550

RAW SEQUENCE LISTING

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:50

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

```

192 agctccctcg tgcgctctcc tgttccgacc ctgccgctta ccggatacct gtccgccttt 4610
193 ctcccttcgg gaagcgtggc gctttctcaa tgctcacgct gtaggtatct cagttcgggtg 4670
194 taggtcggtc gctccaagct gggctgtgtg cacgaacccc ccgttcagcc cgaccgctgc 4730
195 gccttatccg gtaactatcg tcttgagtcc aacccggtaa gacacgactt atcgccactg 4790
196 gcagcagcca ctggtaacag gattagcaga gcgaggtagt taggcgggtg tacagagttc 4850
197 ttgaagtggg ggcctaacta cggctacact agaaggacag tatttggtat ctgcgctctg 4910
198 ctgaagccag ttaccttcgg aaaaagagtt ggtagctctt gatccggcaa acaaacacc 4970
199 gctggtagcg gtggtttttt tgtttgcaag cagcagatta cgcgcagaaa aaaaggatct 5030
200 caagaagatc ctttgatctt ttctacgggg tctgacgctc agtggaaacg aaactcacgt 5090
201 taagggtatt tggatcatgag attatcaaaa aggatcttca cctagatcct tttaaattaa 5150
202 aaatgaagtt ttaaataaat ctaaagtata tatgagtaaa cttggtctga cagttaccaa 5210
203 tgcttaatac gtgaggcacc tatctcagcg atctgtctat ttcgttcac catagttgcc 5270
204 tgactccccg tctgttagat aactacgata cgggaggggt taccatctgg cccagtgct 5330
205 gcaatgatac cgcgagaccc acgctcacgg gctccagatt tatcagcaat aaaccagcca 5390
206 gccggaaggg ccgagcgcag aagtggctct gcaactttat ccgcctccat ccagtctatt 5450
207 aattgttgcc ggggaagctag agtaagtagt tcgccagtta atagtttgcg caacgttggt 5510
208 gccattgcta caggcatcgt ggtgtcacgc tcgtcggttg gtatggcttc attcagctcc 5570
209 ggttcccaac gatcaaggcg agttacatga tcccccatgt tgtgcaaaaa agcgggttagc 5630
210 tcttcgggtc ctccgatcgt tgtcagaagt aagttggccg cagtgttata actcatggtt 5690
211 atggcagcac tgcataattc tcttactgtc atgccatccg taagatgctt ttctgtgact 5750
212 ggtgagtact caaccaagtc attctgagaa tagtgtatgc ggcgaccgag ttgctcttgc 5810
213 ccggcgtcaa tacgggataa taccgcgcca catagcagaa ctttaaaagt gtcatacatt 5870
214 ggaaaacggt cttcggggcg aaaactctca aggatcttac cgtgttgtag atccagttcg 5930
215 atgtaaccca ctctgcacc caactgatct tcagcatctt ttactttcac cagcgtttct 5990
216 ggggtgagcaa aaacaggaag gcaaaaatgcc gcaaaaaagg gaataagggc gacacggaaa 6050
217 tggtgaatac tcatactctt cttttttcaa tattattgaa gcatttatca gggttattgt 6110
218 ctcatgagcg gatacatatt tgaatgtatt tagaaaaata aacaaatagg ggttccgcgc 6170
219 acatttcccc gaaaagtgcc acctgacgtc 6200

```

221 <210> SEQ ID NO: 2

222 <211> LENGTH: 650

223 <212> TYPE: PRT

224 <213> ORGANISM: Homo sapiens

225 <400> SEQUENCE: 2

```

226 Met Arg Pro Leu Arg Pro Arg Ala Ala Leu Leu Ala Leu Leu Ala Ser
227 1 5 10 15
228 Leu Leu Ala Ala Pro Pro Val Ala Pro Ala Glu Ala Pro His Leu Val
229 20 25 30
230 His Val Asp Ala Ala Arg Ala Leu Trp Pro Leu Arg Arg Phe Trp Arg
231 35 40 45
232 Ser Thr Gly Phe Cys Pro Pro Leu Pro His Ser Gln Ala Asp Gln Tyr
233 50 55 60
234 Val Leu Ser Trp Asp Gln Gln Leu Asn Leu Ala Tyr Val Gly Ala Val
235 65 70 75 80
236 Pro His Arg Gly Ile Lys Gln Val Arg Thr His Trp Leu Leu Glu Leu
237 85 90 95
238 Val Thr Thr Arg Gly Ser Thr Gly Arg Gly Leu Ser Tyr Asn Phe Thr
239 100 105 110
240 His Leu Asp Gly Tyr Leu Asp Leu Leu Arg Glu Asn Gln Leu Gly Phe
241 115 120 125

```

RAW SEQUENCE LISTING ERROR SUMMARY

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:51

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw

Invalid Line Length:

The rules require that a line not exceed 72 characters in length. This includes spaces.

Seq#:1; Line(s) 2,49

VERIFICATION SUMMARY

DATE: 08/31/2004

PATENT APPLICATION: US/10/722,371

TIME: 13:37:51

Input Set : N:\Crf3\RULE60\10722371.raw.txt

Output Set: N:\CRF4\08312004\J722371.raw